Spina Bifida Association of America

August 18, 2004

Food and Drug Administration
Division of Dockets (HFA-305)
5630 Fisher's Lane
Rm. 1061
Rockville, MD 20852

Re: Docket 2004N-0254

To Whom It May Concern:

The Spina Bifida Association of America is pleased to submit these comments in response to the Request for Comments on Possible Barriers to the Availability of Medical Devices Intended to Treat or Diagnose Diseases and Conditions that Affect Children.

The Spina Bifida Association of America is the only voluntary health agency that exclusively serves children and adults with spina bifida and their families. The Spina Bifida Association of America is the national voluntary health agency working on behalf of people with Spina Bifida and their families through education, advocacy, research and service. The Association was founded in 1973 to address the needs of the spina bifida community, and today serves as the representative of 57 chapters in more than 125 communities nationwide. In recent years research has become a priority for the Spina Bifida Association of America. Relatively little funding is dedicated to research about spina bifida, its causes and consequences. Specifically, in 2004 a total of only $14M has been dedicated to spina bifida medical research by the federal government.

Spina bifida is a neural tube defect affecting 70,000 people in the United States. It is the most common permanently disabling birth defect and affects 3,000 pregnancies each year. The result of this neural tube defect is that most children with spina bifida suffer from a host of physical, psychological, and educational challenges - including paralysis, developmental delay, numerous surgeries, and hydrocephalus. The challenges associated with spina bifida are such that it is not uncommon for children to have had 20 or more surgeries by age 18. It is estimated that over 80% of people with spina bifida have hydrocephalus, living with a shunt in their skulls which seeks to ameliorate their condition by helping to relieve cranial pressure associated with spinal fluid that does not flow properly.

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The Spina Bifida Association of America is pleased that the Food and Drug Administration (FDA) is seeking to obtain input from all stakeholders pertaining to

- the unmet medical device needs in the pediatric population in the United States;
- ascertaining whether medical device needs are focused in certain medical specialties and/or pediatric subpopulations;
- the possible barriers to the development of new pediatric devices including regulatory hurdles, clinical hindrances, economic issues or legal issues; and
- the steps that the FDA could take to facilitate the development of devices intended for the pediatric population.

Given the needs of people with spina bifida, we will address our comments particularly to devices related to treating hydrocephalus in children and those designed for treatment of children with a neurogenic bladder.

There is no cure for hydrocephalus but in most cases it can be treated effectively with a mechanical implant device to relieve the cerebrospinal fluid. However, according to a 1998 study, fifty percent of shunts will fail in two years. Shunt failure is an all too common problem in children with spina bifida, causing numerous insidious complications, increased hospitalizations and their associated costs, and countless absences from school causing these children, many of whom often live with learning disabilities, to fall further and further behind. While advances in shunt technology have taken place they have represented only small improvements. The problem of minimal improvements in technology is exacerbated by the fact that FDA standards dictate significant requirements involving randomized trials. These stringent requirements are a disincentive to industry to make improvements or to create new products for a relatively small patient population. While this is a concern for the Spina Bifida Association of America and its constituents, the Association is sensitive to and supports maintaining high standards of safety in devices for children with spina bifida.

Similarly, a high priority in urologic treatment and for urologic research is to bring some "normalcy" into the lives of children who live with a common outcome of spina bifida—a neurogenic bladder caused by abnormal innervation. Few satisfactory treatments are available, so a priority in urologic research would focus on abnormal innervation with a treatment that would involve neruomodulation. Unfortunately this type of research is difficult for many reasons, including the high cost and the lack of an animal model to study the effects of such therapy. There are scientists interested in exploring this therapy, but the barriers of little available funding and low motivation on the part of industry to pursue such research for a small population is a frustrating deterrent. Specifically, industry is forced to evaluate future innovations against the costs associated with mounting a major research and development initiative in this area. They must look at the cost of R & D against what payers will support.

Given combined concerns of little research funding, a small population, and stringent standards of the FDA, there has been little innovation and few improvements for the care of children with spina bifida. It is clear that increased research in these areas is critical and from such research, reasonable standards can be developed by the FDA in the

treatment of hydrocephalus and the neurogenic bladder. If adequate research was being funded it would be in the patients' interests to wait for the results in order to create critical safety standards. However since adequate research is not currently underway and not currently planned, how can we as a nation say no to results that look promising from research that is underway? Should children with spina bifida continue to lead lives predicated on inferior quality of life when promising technologies and treatments are on the horizon? While the Spina Bifida Association of America is first and always concerned about the safety of the people we represent, we understand the current reality of cost versus quality and wish the FDA to continue reviewing innovations for spina bifida in a careful and thoughtful manner. We ask the FDA, however, to consider a new paradigm to review innovations for products that would affect smaller patient populations.

We respectfully ask the FDA to consider instituting an innovative program that would review devices developed to answer the needs of people with spina bifida and other orphan diseases. If such a program exists, we request the expansion of the program to further accommodate and recognize current economic realities. For people who suffer from conditions or diseases where there is limited or little adequate research, the balance between patient need and the level of risk may be different than it is for others. For people with spina bifida and other orphan diseases and conditions, there is an immediate need for creating safe standards that at the same time streamline the process for review.

The Spina Bifida Association of America stands ready to join the FDA as it explores the notion of such a demonstration or any other program that will increase the likelihood of improving the quality of life for people with spina bifida.

Thank you for opportunity to comment.

Sincerely,

Cindy Brownstein

Cindy Brownstein
Chief Executive Director